Gaucher Disease Agents
(Elelyso, Cerdelga, Cerezyme, VPRIV, and Zavesca)

NOTICE

This policy contains information which is clinical in nature. The policy is not medical advice. The information in this policy is used by Wellmark to make determinations whether medical treatment is covered under the terms of a Wellmark member's health benefit plan. Physicians and other health care providers are responsible for medical advice and treatment. If you have specific health care needs, you should consult an appropriate health care professional. If you would like to request an accessible version of this document, please contact customer service at 800-524-9242.

BENEFIT APPLICATION

Benefit determinations are based on the applicable contract language in effect at the time the services were rendered. Exclusions, limitations or exceptions may apply. Benefits may vary based on contract, and individual member benefits must be verified. Wellmark determines medical necessity only if the benefit exists and no contract exclusions are applicable. This policy may not apply to FEP. Benefits are determined by the Federal Employee Program.

DESCRIPTION

The intent of the Gaucher Disease Agents policy is to ensure appropriate selection of patients for therapy based on product labeling, clinical guidelines and clinical studies while steering utilization to the most cost-effective medication within the therapeutic class. For this program, Elelyso is the preferred product. Coverage for non-preferred products (Cerezyme and VPRIV) is provided based on clinical circumstances that would exclude the use of the preferred product and may be based on previous use of a product. The coverage review process will ascertain situations where a clinical exception can be made. This program applies to all members requesting treatment with a non-preferred product for an indication that is also FDA-approved for the preferred product.

*Cerdelga and Zavesca are excluded from the preferred product requirement.

The indications below including FDA-approved indications and compendial uses are considered a covered benefit provided that all the approval criteria are met and the member has no exclusions to the prescribed therapy.

<u>FDA-Approved Indications</u>

1. Elelyso is indicated for the treatment of patients with a confirmed diagnosis of type 1 Gaucher disease.
2. VPRIV is indicated for long-term enzyme replacement therapy (ERT) for patients with type 1 Gaucher disease.
3. Cerezyme is indicated for long-term enzyme replacement therapy (ERT) for pediatric and adult patients with a confirmed diagnosis of type 1 Gaucher disease that results in one or more of the following conditions: anemia, thrombocytopenia, bone disease, hepatomegaly, or splenomegaly.

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4. Cerdelga is indicated for the long-term treatment of adult patients with Gaucher disease type 1 who are CYP2D6 extensive metabolizers, intermediate metabolizers, or poor metabolizers as detected by an FDA-cleared test.
5. Zavesca is indicated as monotherapy for the treatment of adult patients with mild to moderate type 1 Gaucher disease for whom enzyme replacement therapy is not a therapeutic option (e.g. due to allergy, hypersensitivity, or poor venous access).

Compendial Uses
Gaucher disease type 3 (Cerezyme, Elelyso, and VPRIV only)

Limitations of Use
Patients who are CYP2D6 ultra-rapid metabolizers may not achieve adequate concentrations of Cerdelga to achieve a therapeutic effect. A specific dosage cannot be recommended for those patients whose CYP2D6 genotype cannot be determined (indeterminate metabolizers).

Table. Gaucher Disease Agents

Medication	Generic Name
Preferred Products:	
Elelyso	taliglucerase alfa
Targeted Products:	
Cerezyme	imiglucerase
VPRIV	velaglucerase alfa

POLICY

Required Documentation
Submission of the following information is necessary to initiate the prior authorization review:
I. Beta-glucocerebrosidase enzyme assay or genetic testing results supporting diagnosis
II. The results of the CYP2D6 test (Cerdelga only)

Criteria for Initial Approval
I. Elelyso is considered **medically necessary** for treatment of Gaucher disease type 1 when the following criteria are met:
   a. Diagnosis of Gaucher disease type 1 was confirmed by enzyme assay demonstrating a deficiency of beta-glucocerebrosidase (glucosidase) enzyme activity or by genetic testing

Approval is for **12 months**

II. Elelyso is considered **medically necessary** for treatment of Gaucher disease type 3 when the following criteria are met:
   a. Diagnosis of Gaucher disease type 3 was confirmed by enzyme assay demonstrating a deficiency of beta-glucocerebrosidase (glucosidase) enzyme activity or by genetic testing

Approval is for **12 months**

III. VPRIV is considered **medically necessary** for treatment of Gaucher disease type 1 when the following criteria are met:
   a. Diagnosis of Gaucher disease type 1 was confirmed by enzyme assay demonstrating a deficiency of beta-glucocerebrosidase (glucosidase) enzyme activity or by genetic testing

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b. Member is currently receiving treatment with VPRIV (excluding when obtained as samples or via manufacturer's patient assistance programs) **OR** member has had a documented inadequate response or an intolerable adverse event with the preferred product Elelyso.

Approval is for **12 months**

IV. VPRIV is considered **medically necessary** for treatment of Gaucher disease type 3 when the following criteria are met:
   a. Diagnosis of Gaucher disease type 3 was confirmed by enzyme assay demonstrating a deficiency of beta-glucocerebrosidase (glucosidase) enzyme activity or by genetic testing

Approval is for **12 months**

V. Cerezyme is considered **medically necessary** for treatment of Gaucher disease type 1 when the following criteria is met:
   a. Diagnosis of Gaucher disease type 1 was confirmed by enzyme assay demonstrating a deficiency of beta-glucocerebrosidase (glucosidase) enzyme activity or by genetic testing
   b. Member is currently receiving treatment with Cerezyme (excluding when obtained as samples or via manufacturer's patient assistance programs) OR member has had a documented inadequate response or an intolerable adverse event with the preferred product Elelyso.

Approval is for **12 months**

VI. Cerezyme is considered **medically necessary** for treatment of Gaucher disease type 3 when the following criteria is met:
   a. Diagnosis of Gaucher disease type 3 was confirmed by enzyme assay demonstrating a deficiency of beta-glucocerebrosidase (glucosidase) enzyme activity or by genetic testing and the member is experiencing neurological symptoms

Approval is for **12 months**

VII. Cerdelga is considered **medically necessary** for treatment of Gaucher disease type 1 when the following criteria are met:
   a. Diagnosis of Gaucher disease was confirmed by enzyme assay demonstrating a deficiency of beta-glucocerebrosidase (glucosidase) enzyme activity or by genetic testing
   b. Member is a CYP2D6 extensive metabolizer, an intermediate metabolizer, or a poor metabolizer as detected by an FDA-cleared test

Approval is for **12 months**

VIII. Zavesca (miglustat) is considered **medically necessary** for treatment of Gaucher disease type 1when the following criteria is met:
   a. Diagnosis of Gaucher disease type 1 was confirmed by enzyme assay demonstrating a deficiency of beta-glucocerebrosidase (glucosidase) enzyme activity or by genetic testing
   b. The member has a documented inadequate response or intolerable adverse events with enzyme replacement therapy.

Approval is for **12 months**

<u>Continuation of Therapy</u>

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I. Cerezyme, Elelyso, and VPRIV are considered **medically necessary** for continuation of treatment of Gaucher disease type 1 or type 3 in members who are not experiencing an inadequate response or any intolerable adverse events from therapy.

Approval is for **12 months**

II. Cerdelga and Zavesca are considered **medically necessary** for continuation of treatment of Gaucher disease type 1 in members who are not experiencing an inadequate response or any intolerable adverse events from therapy.

Approval is for **12 months**

Elelyso, VPRIV, Cerezyme, Cerdelga, and Zavesca are considered **not medically necessary** for patients who do not meet the criteria set forth above.

Dosing and Administration
Approvals may be subject to dosing limits in accordance with FDA-approved labeling, accepted compendia, and/or evidence-based practice guidelines.

PROCEDURES AND BILLING CODES

To report provider services, use appropriate CPT* codes, Alpha Numeric (HCPCS level 2) codes, Revenue codes, and/or ICD diagnostic codes.

- J3060 – Inj, taliglucerace alfa 10 units (Elelyso)
- J1786 – Inj, imiglucerase, 10 units (Cerezyme)
- J3385 – Inj, velaglucerase alfa, 100 units (VPRIV)

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POLICY HISTORY

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